THE
PATIENTS' COALITION

An Independent Coalition of Patients With Serious And Life-Threatening Diseases Working Together For Responsible FDA Reform

"The Prescription Drug User Fee Act and Reform of the Food and Drug Administration"

TESTIMONY BY JEFF BLOOM
FOR THE PATIENTS' COALITION

BEFORE

THE COMMITTEE ON COMMERCE
SUB-COMMITTEE ON HEALTH AND THE ENVIRONMENT
UNITED STATES HOUSE OF REPRESENTATIVES

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SUMMARY OF TESTIMONY

1. The American patients' interest in the FDA reform debate.

2. Patients' Coalition poll results verifying broad support among American voters for the Food and Drug Administration.

3. The Congress must revise the Prescription Drug User Fee Act swiftly without attaching contentious FDA "reform" proposals.

4. The budget for FDA must be sufficient for FDA to fulfill its mission of promoting and protecting the health of American consumers.

5. Legislation should be limited to the expansion of the 1-800 TRIALS-A and AIDS Clinical Trials Information Service to all patients with serious or life-threatening illness; increasing sunshine on the drug application and approval process, and the expansion of enforcement mechanisms available to the FDA to fulfill its mission.

6. The Patients' Coalition believes deregulating off-label dissemination of information presents a direct threat to the nation's public health.

7. Improving the supplemental approval process is the correct way to provide information about new uses of drugs.

8. The Patients' Coalition does not support any action that would lower the standards of safety and

effectiveness for drug approvals.

Good afternoon, Mr. Chairman, and members of the Subcommittee.

My name is Jeff Bloom and I am a volunteer Legislative Representative with Project Inform, one of the nation's leading AIDS information and advocacy organizations. I appear today on behalf of the Patients' Coalition, a group of over 100 organizations representing patients with serious and life-threatening illnesses and consumers who depend on the FDA to guarantee the safety and effectiveness of our nation's drug supply. Neither Project Inform or the Patients' Coalition receive or have received any federal grants or contracts for the past two years.

The Patients' Coalition came together two years ago in response to a flood of disturbing and misleading rhetoric concerning the future roles and responsibilities of the FDA. Many of our member groups were bombarded with messages such as "The Patient is Waiting," although no patient group with whom we are associated was being asked for their input into early reform proposals. Many of the charter members of the Patients' Coalition represented women, AIDS patients, people with rare disorders and senior citizens and, so, had compiled a long history of activism on issues around drug regulation and development. Many of our groups have actively engaged in forcing changes at the FDA and, therefore, had become educated about what works and doesn't work at the FDA. As the debate has moved forward, it became ever more clear that the real concerns of patients are not included in the agendas of those who would "reform" the FDA.

Over the past few years, Congress has been subject to countless attempts by members of the regulated industries to explain what patients needed from the FDA. Our frustration arose from the complete lack of reliable data suggesting that the present system withheld access to important drugs from American patients. There was, in fact, ample data suggesting that the FDA's drug approval

processes have successfully promoted and protected America's public health while speeding access to lifesaving therapies.

A recent poll commissioned by the Patients' Coalition (appended) verified that the American public placed great trust in the FDA. Eighty-seven percent (87%) of voters stated that the FDA was necessary. Support for the FDA also cut across party lines with 86% of Republicans, 88% of independents and 88% of Democrats stating that the FDA was necessary_. Sixty-seven percent (67%) of respondents stated that they trusted the FDA more than the pharmaceutical industry to make sure that medicines and drugs were safe, while a mere 13% of the respondents trusted the drug companies more than the FDA. Sixty-three percent (63%) of Republicans stated that they trusted the FDA more than the drug companies while only 15% of Republicans trusted the drug manufacturers more. The argument that the FDA is a "big government bureaucracy" drew only shallow support (22% agreed) compared to those who felt that the FDA was necessary to protect the public (68%). Even among those who identified themselves as conservative Republicans, only 25% agreed more with the bureaucracy argument while 65% agreed that the FDA effectively protects the public.

Finally, in a question regarding the need for greater FDA enforcement mechanisms, an overwhelming 82% of voters supported granting the FDA the power to fine drug manufacturers who failed to conduct additional research on drugs when required to by the FDA. These poll results clearly show broad and bipartisan support for a strong FDA that protects the health and safety of all Americans.

Our central message today is that Congress must revise and extend the Prescription Drug User Fee Act (PDUFA) swiftly without extraneous amendments or legislative riders. We understand that there

are ongoing discussions between the industry and the FDA to determine appropriate changes to PDUFA prior to reauthorization. We support prudent changes to improve this program. We firmly believe, however, that it is critical that all stakeholders — including patients — be actively involved in determining what those changes will be. Once PDUFA II is agreed upon, the reauthorization process should move forward with all deliberate speed.

The move to link FDA reform legislation to the reauthorization of this vital program is, we believe, short-sighted and self-defeating. It is completely inappropriate to link these two pieces of legislation for a number of important reasons. First, linking contentious FDA reform proposals to PDUFA reauthorization threatens the future vitality of a program that has benefitted hundreds of thousands of American patients. The FDA has met performance goals agreed on under PDUFA I in less time than was called for under the statute. These key goals were met because PDUFA provided the FDA with the additional resources necessary to improve the drug approval process without changes that could have harmed the FDA's ability to adequately determine safety and efficacy. PDUFA is FDA reform that is proven to work. Rather than threaten the reauthorization of PDUFA we should congratulate the wise drafters of such a remarkably effective statute, immediately reauthorize the legislation, and move on. PDUFA must not be held hostage to FDA "reforms"; the need for which a consensus has yet to be built. No proposed "reforms" will have as important an effect on the ability of the FDA to process applications as does reauthorization of PDUFA itself. In drug approval terms, the risk/benefit ratio is far too risky.

Our understanding is that the FDA will have to begin dismantling PDUFA by this summer if the program is not reauthorized. This threat is unacceptable to patients. This threat is also unacceptable to the industry. This threat should also be unacceptable to the members of this subcommittee and to Congress.

If the FDA is to remain an effective force for the public health, the important question of FDA resources must be addressed. Much as there is a irreducible minimum time needed by the FDA to ensure that drugs and devices are safe and effective, there is also an irreducible level of resources the FDA must have to do its job. Under the President's proposed FY 1998 budget, the FDA is being asked to do much, much more with much, much less.

The Administration's budget request includes funding levels which rely on medical device and other user fees to cover a large portion of the FDA's base budget. Unfortunately, the Agency has no authority to collect such fees nor is it likely to gain such authority in the near future. Even if these fees were implemented, however, the Administration's proposal would allocate them to finance core program and public health functions at the FDA and not to create enhancements to those functions that would justify the added fiscal burden the fees impose on regulated industries. As your colleagues in the House Agriculture Appropriations Subcommittee have made clear, this budgetary tactic seems predicated on the Congress's strong desire to maintain the public health function of the FDA to provide cover for the Administration's claims regarding balanced budgets. The Patient's Coalition will strongly oppose any attempt to balance the budget at the expense of weakening the FDA.

As to the question of whether legislation is required to "reform" the FDA, The Patients' Coalition believes that there are limited areas that could be addressed by Congress that would protect rather than to alter or subvert the core mission of the FDA. Those limited areas include medical device and generic drug user fees, enhanced enforcement authority including civil monetary penalties/injunctive relief, and expansion of the clinical trials information service modeled on the current AIDS clinical trials database.

There are also administrative steps the FDA could take that would lead to more efficient reviews and approvals of safe and effective drugs and devices, that would maintain a safe and wholesome food supply, and would guarantee greater public access to crucial health-related information. We must emphasize, however, that these important steps could be accomplished without legislation.

We fought hard last year against many "reform" proposals because they would have undermined the fundamental authority of the FDA. We feel just as adamantly today that FDA reform legislation is unnecessary and would weaken the Gold Standard set forth by the Food, Drug, and Cosmetic Act. In truth, proposed legislative reforms seemed little more than a concerted attempt to lower the standards by which drugs are tested, approved, and marketed in this country. Many of the arguments for these measures have been based on half-truths and distortions. As organizations representing people with serious and life-threatening diseases, we firmly support and have worked to assure that patients have access to new and possibly life-extending therapies. We also firmly believe, however, that it is in the best interest of all patients and consumers for the country to maintain the highest possible drug research and approval standards. Unfortunately, we remain convinced that any FDA reform legislation introduced in this Congress will likely include provisions that will those standards.

There are a number of areas where the effect of reform may be to lower the nation's drug approval standards. The most threatening of these areas to patients are proposals to substantially deregulate the promotion of drugs for uses that not been proven safe or effective. Such off-label promotion is a hazard to the American public and presents a direct threat to the nation's public health. The purpose of the Food, Drug and Cosmetic Act (FDC&A) is to safeguard the public health -- not to permit marketing campaigns for unapproved drugs by an already enviably profitable industry. Allowing companies to promote uses that have never been proven safe and effective would undercut the fundamental premise upon which the nation's drug approval system is built.

We realize that off-label **use** is very common; especially in the treatment of cancer, a disease where experimental treatments of all kinds are regularly employed. We also support the right of informed physicians to prescribe approved drugs for unapproved uses. We strenuously oppose, however, the promotion of such uses by manufacturers. Relaxing the standards related to the dissemination of such information poses a substantial threat to future research on product uses and the filing of supplemental applications. Deregulating off-label promotion would allow companies to take advantage of the benefits of an approved indication without ever scientifically proving the indication's safety and effectiveness. Thus, there would be no incentive for companies to conduct the research to prove additional health claims. At the same time, allowing off-label promotion penalizes those companies that do the important clinical research necessary to prove indications.

In addition to the serious safety and effectiveness issues raised by off-label promotion, an all-too-real pocketbook issue for many patients is reimbursement by insurance companies for off-label prescription drugs. Insurance companies often refuse to pay for drugs prescribed for non-FDA approved indications. The solution to the reimbursement problem, as well as to improving patients and doctors access to accurate clinical information, is to encourage widespread use of the supplemental approval process, not to allow unrestrained marketing of unproven uses.

Make no mistake, women, children, and people living with serious and life-threatening diseases would bear the brunt of such a dangerous change. Accurate clinical information would be less likely to be collected on the proper use of drugs marketed to these populations. For example, after many years spent fighting to increase the level of health research to benefit these populations, companies continue to enroll fewer women than men in clinical trials, and do little to collect data on clinical experiences unique to women. Additionally, children are not simply little adults. children, still

growing and changing, react to drugs and devices differently, and we cannot assume that what is safe for adults is safe for children.

There has been much interest in the difficulty that pediatric patients have in gaining indications for pediatric formulations of drugs. It would seem, therefore, that an institution as well respected as the American Academy of Pediatrics (AAP) would support off-label promotion. In a letter submitted to the Senate Labor and Human Resources Committee, AAP stated that it does not support off-label promotion for many of the same reasons we outline above.

The opportunity for companies to act irresponsibly would be far too great if off-label promotion by the industry were allowed. The number of drugs prescribed for off-label uses which have hurt consumers are almost too numerous to count. There are compelling examples:

- Clonidine, a high blood pressure drug, was prescribed more than 200,000 times in 1994 to treat children with attention deficit disorders. Research has discovered severe side effects and a handful of deaths.

- Ambocor, Enkaid and similar drugs for irregular heartbeats were widely prescribed off-label in hopes they would prevent a lethal electrical breakdown of the heart. Government sponsored testing later proved that these drugs caused cardiac arrest rather than preventing it. Tens of thousands of patients died prematurely.

- Tamoxifen and Lupron are two drugs prescribed off-label to women with fibrocystic breasts to reduce the periodic pain associated with the condition. Both these drugs are known to cause serious side effects; tamoxifen causes cancer of the uterus and blood clots while

Lupron produces severe bone loss while it is used.

There are preferable ways to speed to patients and consumers balanced, scientifically accurate information free from the taint of industry influence. One proposal would require the Department of Human Services to expand the current clinical trials information service benefiting people with AIDS to all patients with serious or life-threatening diseases. 1-800-TRIALS-A offers people with HIV/AIDS the ability to participate in clinical trials and receive early access to potentially life enhancing new therapies. Additionally, the AIDSDRUG database provides accurate and **complete** information about AIDS drugs to patients and their doctors, families, and friends.

Senator Olympia Snowe and Senator Dianne Feinstein have taken the lead on this initiative by introducing legislation to expand 1-800-TRIALS-A and the AIDSDRUG service to all people with serious and life-threatening diseases through the Public Health Service Act. These services provide complete and accurate information to patients and clinicians on experimental drugs, clinical trials, and approved therapies.

Regarding so-called "third-party review," a laudable consensus emerged last year that any use of outside parties should be firmly placed under the control of the FDA. We support that consensus. Any other arrangement would threaten the integrity of the regulatory process and the health and safety of the American public.

Additionally, we support a number of pro-active changes which the FDA, working with patient and consumers and the regulated industries, could adopt without sweeping legislation.

First, the Patient's Coalition strongly supports the FDA's ability to speed the approval of new therapies to patients with few other options. However, pressures imposed by the regulated industries have often makes the FDA hesitant to release public information about the status of applications and petitions. While we understand the need to protect proprietary data, it is important that consumers have as much access as possible to information regarding the progress of applications and petitions. Such sunshine will create mutual responsibility between the FDA and industry, effectively producing higher quality applications, more efficient reviews, and, ultimately, better drugs and devices.

Second, women, children and people of color have been largely absent or excluded from participation in clinical trials resulting in very little data about the short or long-term effects of drugs on these populations. Especially in the case of FDA's fast-track approval of drugs for serious and life-threatening illnesses based on limited clinical trial data, follow up studies to confirm safety and effectiveness are critical. The Patient's Coalition believes that the agency must take have the tools, such as civil monetary penalties, to compel drug sponsors to perform all appropriate post-marketing studies to insure safety and effectiveness for patients.

Third, the Prescription Drug User Fee Act has worked effectively to improve the drug approval process and has the support of the FDA, consumers, and the regulated industries. Many of the current problems raised with respect to the device review process could be resolved more efficiently and effectively with the adoption of a similar user fee program.

Finally, we believe that insufficient attention has been paid to reforms initiated by both the FDA and by the Vice-Presidents Reinventing Government Initiative. The Patient's Coalition has been impressed by the FDA's efforts to identify -- with the help of the regulated

industries, patients, consumers and other interested parties -- solutions to those problems which may have hampered the Agency's efficiency. These "reforms", have made great strides in speeding safe and effective products to the public. This progress is particularly clear in the case of New Molecular Entities (NMEs) developed to treat cancer, AIDS, Alzheimer's Disease, and multiple sclerosis. In fact, the GAO has reported that, in a comparison of 15 new drugs approved both by the FDA and the EU centralized procedure, Americans had earlier access to the drugs than did Europeans. According to the January 1997 issue of Scrip Magazine, more pharmaceutical companies chose the United States in 1996 for the introduction of their NMEs into market than any other country in the world. It clearly follows, then, that the FDA must be doing something right.

The issues we've discussed this afternoon are closely linked. If the budget problem is resolved and PDUFA is reauthorized but the end result is the adoption of harmful legislation that lowers the nation's drug approval standards, then we are in a worse position than we are in today. We do not support PDUFA reauthorization if the cost is the weakening of the FDA drug approval process.

For the hundreds of thousands of patients and consumers represented by the Patients' Coalition nothing is more important than access to safe and effective life-extending therapies. We owe it to these patients and their families to protect and promote public health through a strong, well-resourced and fully-funded Food and Drug Administration.

We urge you and the entire Subcommittee to ensure a strong and effective FDA. Thank you once again for the opportunity to speak with you today, and I will be happy to answer any questions.

BIOGRAPHY

Jeff Bloom is the Washington D.C. volunteer legislative representative with Project Inform, one of the nation's leading AIDS information and advocacy organizations. Before joining Project Inform, Mr. Bloom served as volunteer FDA Fellow at AIDS Action and as a Treatment and Research volunteer with the National Association of People with AIDS.

A long-term survivor of AIDS, Mr. Bloom attended the University of Maryland at College Park. After graduating in 1981, Mr. Bloom embarked on a career in business including over 10 years as Chief Executive Officer and Chief Financial Officer of a multi-million dollar audio-video corporation. Complications from HIV infection of the spinal cord caused Mr. Bloom to retire from corporate life in August 1994 and embark on his current path of volunteerism.

In addition to his work as legislative representative with Project Inform, Mr. Bloom presently serves on the board of TIICANN - The Title II Community AIDS National Network. In addition, he is member of the Georgetown University AIDS Clinical Trials Advisory Board and represents Project Inform on the legislative affairs committee of the AIDS Drug Assistance Program Working Group and The Patients' Coalition for Responsible FDA Reform.